



October 22, 2014

The Honorable John Austin
Chairman, Washington State Board of Health
P.O. Box 47990
Olympia, WA 98504-7990

Re: Newborn Screening for Adrenoleukodystrophy (ALD) in Washington State

Dear Mr. Austin:

Following my public testimony on April 9, 2014 at the State Board of Health meeting, our family foundation was very pleased to receive your letter dated April 11, 2014, in which you indicated that the Board had accepted our recommendation to convene a newborn screening advisory committee to review ALD for potential inclusion on the state's mandatory newborn screening panel.

Since receiving your letter, Ms. Tara Wolff, Health Policy Advisor for the Board has kept me apprised of the initial steps that have taken place since forming the advisory committee. Specifically, it is my understanding that the advisory committee has conducted two meetings. Following the initial meeting, Dr. John Thompson of the Washington State Newborn Screening Laboratory was tasked with drafting a Preliminary Reference Search on ALD, which was subsequently reviewed with the advisory committee members at the second meeting. It is also my understanding that during the second meeting, the advisory committee had discussed the possibility of suspending further efforts in its ongoing evaluation of ALD in the absence of any measurable progress in the federal government's advancement of adding ALD to the Recommended Uniform Newborn Screening Panel (RUSP).

Under Health and Human Services (HHS), the Discretionary Advisory Committee on Heritable Disorders in Newborns and Children (DACHDNC) unanimously voted in January 2014 to advance ALD to external evidence review – the final step before making a formal recommendation to the Secretary of HHS to add new conditions to the RUSP. Unfortunately, the external evidence review process for ALD has been delayed for the past nine months due to administrative issues related to contract expirations with members of the external evidence review committee. Although we

have received recent confirmation from DACHDNC that all contracts have since been renewed and external evidence review for ALD is scheduled to commence before the end of this year, we expect it will be at least another 18-24 months before a formal recommendation is submitted to the Secretary of HHS to add ALD to the RUSP.

In order to avoid the situation where Washington State's ongoing analysis and evaluation of ALD would be suspended until such future date that ALD is officially added to the RUSP, we wish to formally submit the following proposal:

The Zakes Foundation requests that the State Board of Health commission the advisory committee to continue its full evaluation of ALD for potential inclusion on Washington State's mandatory newborn screening panel. In the event the advisory committee recommends and the State Board of Health votes in approval of adding ALD to the list of conditions to be screened, then the implementation of any future testing in Washington State would be conditioned upon the inclusion of ALD to the RUSP at the federal level.

We believe that this proposal makes good sense for the following reasons:

First, we recognize that the Washington State Board of Health has traditionally waited until conditions are added to the RUSP, before taking action to add any new diseases to the state's mandatory newborn screening panel. Doing so provides the state with additional validation and allows Washington to leverage the work done at the federal level. However, by working in parallel, we gain the benefit of completing the state-level analysis and being implementation-ready, rather than waiting until ALD has been added to the RUSP (possibly as long as 18-24 months from now) before initiating the required state-level evaluation.

This is similar to what has now been successfully accomplished in three different states. Connecticut, New Jersey and most recently California have all passed legislation requiring that ALD be added to their respective mandatory newborn screening panels with the provision that actual testing will not be implemented until ALD is officially added to the RUSP. Thus, consistent with our proposal, each of these states has completed their state-level analyses and are now effectively implementation-ready pending inclusion of ALD to the RUSP.

Second, young boys will continue to die every year from ALD in Washington State as a result of not having the benefit of early detection and diagnosis. For those boys that develop the lethal childhood cerebral form of ALD, stem cell transplant therapy has been shown to be a highly effective treatment. Regrettably, the majority of these young boys will not have the benefit of receiving this life-saving therapy simply because they will not have been diagnosed in time. Tragically, nearly all of these boys will suffer a rapid neurological decline, leading to a vegetative state and ultimately death.

Therefore, every day that we can compress the timeframe for implementation of ALD newborn screening offers the potential to save young boys' lives. By continuing its evaluation of ALD in parallel with the work that is being done at the federal level, Washington State can achieve implementation readiness and be prepared to initiate testing as soon as ALD is added to the RUSP.

New York is the first state to have begun actual testing for ALD. Screening commenced in January 2014. In the first seven months of testing, six ALD cases were positively identified, affording these young babies with the benefit of early diagnosis. Five additional cases of Peroxisomal Biogenesis Disorder (PBD) were also positively identified. There have been no false positive results reported to date. As a result of early diagnosis, each of these babies can now be carefully monitored and effective life-saving therapy can be administered at the appropriate timeframe.

In summary, an accurate and reliable newborn screening method has been developed for the detection of ALD. New York is currently screening babies for ALD with encouraging results. Connecticut, New Jersey and California have each passed legislation mandating that newborn screening for ALD go into effect once ALD is officially added to the RUSP. Under this proposal, we request that Washington State do the same. We strongly encourage the State Board of Health to commission the advisory committee to initiate a full evaluation and to make a formal recommendation to the Board regarding the potential inclusion of ALD to the state's mandatory newborn screening panel. We request that this process occur in parallel with the ongoing work that is being performed at the federal level. If, upon completion of this evaluation, the advisory committee recommends adding ALD to the state's mandatory newborn screening panel and the State Board of Health votes in approval, then under this proposal, actual testing would not commence until such future date that ALD is officially added to the RUSP.

I would be happy to meet with members of the advisory committee to discuss the specifics of this proposal if that would be viewed as helpful.

Thank you in advance for your consideration of this proposal,

Sincerely,



Bradford A. Zakes
President, The Zakes Foundation
18116 232nd Avenue NE
Woodinville, WA 98077
Phone: 425-949-8455